HOUSE OF COMMONS LIBRARY

DEBATE PACK

Number CDP-0130, 14 December 2015

Access to specialist neuromuscular care and treatments

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Contents

Summary

This debate pack has been compiled ahead of the debate on Access to specialist neuromuscular care and treatments, to be held on Tuesday 15 December 2015 at 2.30pm in Westminster Hall. The Member in charge of the debate is Cheryl Gillan.

There are more than 60 different types of neuromuscular condition and it is estimated that around 60 to 70,000 people in the UK live with neurological conditions affecting the functioning of the muscles. The debate is expected to focus on muscular dystrophies, such as Duchene muscular dystrophy (other neuromuscular disorders include motor neuron disease, Parkinson's disease and multiple sclerosis). NHS England is responsible for commissioning specialised neurological services, which includes services for patients with neuromuscular disorders. NHS England has published a service specification for neurological care, which sets out what providers must have in place to offer evidence-based, safe and effective services:

www.england.nhs.uk/wp-content/uploads/2013/06/d04-neurosci-spec-neuro.pdf

The NHS England service specification sets out that patients with neuromuscular conditions, such as muscular dystrophy, should have access to a multidisciplinary team (MDT) who will assess, diagnose and provide support. The MDT team will include neuromuscular consultants, neuromuscular physiotherapists, specialist nurses, occupational therapists, speech and language therapists and other care professionals. One of the key service outcomes of the specification is that all patients with long-term neurological conditions have an individual care plan.

1. Press releases

Action Duchenne, December 9th 2015

NICE decision on Translarna: Reasons for optimism?

After two Evaluation Committee Meetings and an exhaustive consideration of statements and evidence from patient organisations, PTC Therapeutics, and clinicians alike, NICE are set to offer their final guidance by the end of next week.

This is a landmark decision and could end the agonising wait for the delivery of this treatment to all those eligible to benefit in England, Wales and Northern Ireland. This is a day Action Duchenne have been working towards for fourteen years, and we are proud to have stood shoulder to shoulder with the whole community in fighting throughout the past year for Translarna's availability.

The initial indications surrounding this decision appear positive. In a recent debate discussing access to emerging treatments in Parliament, the Minister for Life Sciences, George Freeman MP, expressed hope that Translarna could be made available subject to a 'Managed Access Agreement', such as with Vimizim for the treatment of Morquio A. This is an agreement between NICE and the drug manufacturer to initially make the treatment available up to five years, subject to continued data collection, monitoring and a negotiation on price.

Mr Freeman stated, "Following the positive news on Vimizim, I am hopeful about Translarna, a similar drug. […] I am hopeful that there will be a decision in the next few months to parallel the one on Vimizim, but that decision is not in my gift: it is up to NICE".

Action Duchenne have been considering this as an eventuality and are currently working with PTC Therapeutics on the creation of a Managed Access Document to form the basis of an agreement with NICE over the short-medium term availability of the treatment. It is vitally important that we are ready to respond to any decision made by NICE to help deliver this treatment to families as quickly as possible.

Accordingly, we are also doing everything we can to prepare for all other eventualities, for whilst there appears to be cause for cautious optimism about the forthcoming decision next week, it would be foolish for us to assume that the fight for Translarna is over. Indeed, whilst next weeks statement will offer guidance, the policy is not scheduled to be signed off by NICE until next February. Patients and families have

already been waiting far too long for this treatment to become available. We do not have any more time to wait.

If NICE offer positive guidance, we will do everything we can to ensure the NHS is directed to release funds immediately. Conversely, if NICE offer negative guidance then we will do whatever we can to guarantee the decision is overturned by February and ensure that Translarna is funded when the policy is signed off.

National Institute for Health and Care Excellence (NICE), 15 October 2015

NICE asks for further evidence for the benefits of a new treatment for Duchenne muscular dystrophy to justify its very high cost

In draft guidance produced by its Highly Specialised Technologies programme, NICE has asked for further clarification from PTC Therapeutics on the size of the benefit its drug ataluren (Translarna) provides in the treatment of a type of Duchenne muscular dystrophy (DMD). NICE has asked the company to provide the results of a confirmatory study of ataluren as soon as possible. The draft guidance also asks the company to provide further justification for the cost of the drug.

DMD is one of a group of muscular dystrophies. These are inherited genetic conditions that cause the body to produce too little dystrophin, a substance crucial for muscle functioning. This leads to changes in the muscle fibres which gradually weaken the muscles, resulting in an increasing level of disability. The decline in physical functioning in DMD leads to respiratory and cardiac failure and eventual death, usually before the age of 30.

The draft guidance relates to people with DMD with a mutation (known as a nonsense mutation) in the dystrophin gene. Around 8–13 boys are born with the condition each year in the UK.

Current treatment options for patients with DMD in England are limited and aim to alleviate symptoms and manage complications. These include cardiac and respiratory monitoring and support, orthopaedic intervention and spinal surgery. The current mainstay of treatment, corticosteroid therapy, can cause serious unwanted effects such as growth retardation, bone thinning, mood swings and weight gain.

Ataluren is the first licensed treatment for DMD that addresses the loss of dystrophin, the underlying cause of the condition. It has a conditional marketing authorisation in the UK for the treatment of DMD resulting from a nonsense mutation in the dystrophin gene, in patients who are able to walk aged 5 years and older.

Commenting on the draft guidance, **Professor Carole Longson, NICE Health Technology Evaluation Centre Director, said:** "We are disappointed not to be able to recommend ataluren in this draft guidance. DMD is one of the most common and severe forms of muscular dystrophy. It reduces life expectancy and causes debilitating symptoms that severely affect the quality of life of people with the condition, and their families and carers.

"The Committee heard from the patient experts that one of the most important aspects of managing DMD is maintaining their child's ability to walk because this means they can continue to go to school independently and participate more fully in social and sporting activities with family and friends. There is, therefore, a clear need for a treatment that can prolong a child's ability to walk but without serious side-effects.

"After considering the evidence, and the opinions of the clinical and patient experts, the Committee agreed that ataluren represents an important development in the treatment of DMD and could potentially prolong the time before children have to use a wheelchair, compared with best supportive care. However, the Committee was not convinced that the proposed cost of ataluren was justified by the evidence presented on the additional health benefits associated with ataluren over standard therapy. Therefore, on the basis of the current evidence, the Committee was minded not to recommend ataluren for treating nonsense mutation DMD."

The Committee was made aware that the results of a confirmatory study of ataluren (PTC124 GD 020 DMD; Study 020) are due to be available shortly. The draft guidance therefore indicates that the results of this study should be presented to NICE when the company submits further clarification of the size of benefit ataluren provides for patients, carers and family members, and further justification of the cost of the drug.

NICE has not yet issued final guidance to the NHS; these decisions may change after consultation. Consultees, including the company, healthcare professionals, patient/carer organisations and members of the public are now able to comment on the preliminary recommendations which are available for public consultation until 5pm on 6 November 2015. Comments received during this consultation will be considered by the Committee at its next meeting. This meeting is scheduled for 17 November 2015, but may have to be postponed if the company is unable to provide the data of the ongoing study to NICE in time for the meeting. Following this meeting, the next draft guidance will be issued.

Ends

NHS England, 2 July, 2015

NHS England announces annual investment decisions for certain specialised services

NHS England has today (2 July) set out its planned investment decisions for certain specialised services as part of its annual commissioning round.

The decision follows a three-month public consultation on the principles and processes NHS England will follow when making investment decisions. Feedback from the consultation has helped inform the way these decisions have been made. NHS England has listened to the views of patients and service users and also taken advice from clinical experts and the independently chaired Clinical Priorities Advisory Group (CPAG).

Thirty nine proposed new investments have been carefully considered against the principles NHS England follows when making investment decisions. The principles can be found in Appendix A of NHS England's response to consultation on investing in specialised services.

[…]

NHS England has agreed with the recommendations from CPAG that final funding decisions on the following two treatments should be made after NICE has concluded its Highly Specialised Technology Appraisal process:

- Elosulfase alfa (Vimizim) for Morquio A Syndrome.
- **Ataluren (Translarna) for Duchenne muscular dystrophy**.

[…]

NICE is expected to produce draft guidance on ataluren (Translarna) in October, with final guidance to follow shortly after. The European

medicines regulator has also asked the manufacturer to undertake further studies of the treatment for completion in October.

[…]

James Palmer, NHS England's Clinical Director for Specialised Commissioning, said: "These are really difficult decisions, which is why we rely on NICE wherever possible, and make use of the best available evidence as well as extensive engagement with clinical experts, patient representatives and the public. The new policies we have been able to approve will help us to improve and extend thousands of lives for years to come through prevention, identification and treatment."

Specialised services are those services which are provided from relatively few specialist centres. Conditions treated range from long-term conditions, such as renal (kidney services), to rarer conditions such as uncommon cancers, burn care, medical genetics and specialised services for children.

The clinical commissioning policies considered as part of this process will be made available on the NHS England website from tomorrow (Friday 3 July)

Groups representing patients and services users, and other stakeholders have been told the outcome of the decisions and have been invited to meet with senior NHS England staff where they will have the opportunity to hear in detail the rationale for each decision.

NHS England typically forms clinical commissioning policies as part of the annual round of service commissioning. Where more rapid interim decisions need to be made policies can also be developed through the In Year Service Review policy or through the development of a Policy Statement.

Additions to the work programme for policy development either come from recommendations from the relevant Clinical Reference Group, from the Individual Funding Request process if an intervention is declined as it is part of a 'cohort', or where a Critically Clinically Urgent policy decision has been made.

NHS England clinical policies will be replaced where NICE form either a Technology Appraisal or a Highly Specialised Technology Appraisal. It is the responsibility of provider Trust clinicians to maintain their knowledge of both NICE and NHS England national clinical policy for specialised services so they can inform their patients of treatment choice.

Routes remain open to clinicians for patients with exceptional or critically clinically urgent need, to access a certain treatment or service, even where a clinical commissioning policy has not been approved through this process.

Muscular Dystrophy UK, 10 June 2015

Boys delivered last chance plea to PM as decision looms on breakthrough drug

Six boys with a life-limiting muscle-wasting condition delivered letters to 10 Downing Street earlier today in a last chance bid to appeal for help from the Prime Minister. Their plea comes ahead of a decision being taken in the coming weeks on a breakthrough drug, Translarna, which could enable them to continue walking for longer.

The children's families and Muscular Dystrophy UK have campaigned for NHS England to make Translarna available, since it became the first EU-approved drug for tackling Duchenne muscular dystrophy* last August. The condition affects 2,500 people in the UK, causing increasingly severe disability and cutting short lives.

Translarna could allow the boys to stay on their feet for years longer and is available in Germany, Spain, France, Italy, Denmark, Estonia and Greece since last year. Administrative delays have left UK parents facing an agonising wait for NHS England's decision on the drug.

In quite literally a race against time, boys with Duchenne muscular dystrophy must be able to walk to be eligible for Translarna.

View the six letters by the boys here
A gallery of downloadable photos from today's event is here

Earlier today during PMQs in the House of Commons, Mary Glindon MP challenged the Prime Minister on the issue and called on him to urgently meet the boys visiting Downing Street today. In his response, the Prime Minister recalled meeting Archie Hill, referring to him as 'an amazing young boy—incredibly brave.'

A Downing Street spokesperson said:
Duchenne Muscular Dystrophy is a severe and debilitating condition and the Government completely understands the urgency with which the boys and their families are seeking a decision regarding this drug.

"Decisions on drugs like Translarna must always be taken by clinical experts based on the most up-to-date medical evidence.

"It is absolutely right that children and their parents should ask for the very best from our health service and NHS England are urgently looking at this particular drug and its use."

Sue Barnley from Hampshire, whose son Harry (6) was among the boys, said:

We are very proud of Harry for writing his own letter to the Prime Minister. He absolutely loves football and playing with his brother Jack. As a parent we do all we can to help our children and it's amazing to think that we may have the chance to give them precious years longer on their feet to be able to do the things they love.

"Translarna is the first drug to ever become available for Harry's condition and it's an absolute sham that it has faced so many delays. NHS England has a responsibility to say 'yes' for us and our boys."

Robert Meadowcroft, Chief Executive of Muscular Dystrophy UK, said:

"Translarna is the first and only treatment tackling the cause of Duchenne muscular dystrophy. We simply cannot afford to refuse it to the group of boys whose lives it could transform and we urge Mr Cameron to listen to the desperate please of these and their families.

"It is essential that these boys have immediate access to this drug in the same way that it is already available to boys in other countries across Europe. We find it appalling that these young boys have been denied access in this country to date and now is the time for this to be put right."

*****ENDS*****

* Translarna is the first drug to deal with the underlining genetic cause of Duchenne muscular dystrophy. It has been designed for 10-15 percent of the 2,500 children and adults in the UK who have Duchenne muscular dystrophy caused by a 'nonsense' or 'stop' mutation. Children must be over the age of 5 and to still be walking in order to be eligible for the drug.

2. Press articles

Guardian, 3 December 2015

Parents of boys suffering from muscular dystrophy appeal to NHS to supply drug

Financial Times, September 28, 2015

Patient lobby groups can be a catalyst for new therapies

Health Service Journal, 3 July, 2015

NHS England announces specialised treatment decisions

Health Service Journal (HSJ), April 1, 2015

Exclusive: NHS England labelled 'dysfunctional' over drug policy delay

Health Service Journal (HSJ), February, 2014

Why coordinated care is key for rare disease patients

3. Parliamentary coverage

3.1 PQs

Hydrotherapy

Asked by: Brake, Tom

To ask the Secretary of State for Health, if he will conduct a national review of hydrotherapy provision.

Answering member: George Freeman | Department of Health

NHS England is responsible for commissioning specialised neurological services, which includes services for patients with neuromuscular disorders. NHS England has published a service specification for neurological care, which sets out what providers must have in place to offer evidence-based, safe and effective services. The specification can be found at the following link:

www.england.nhs.uk/wp-content/uploads/2013/06/d04-neurosci-spec-neuro.pdf

Specialised neuromuscular care may include referral to local physiotherapy, hydrotherapy or rehabilitation if appropriate, however the commissioning of hydrotherapy services is a matter for local clinical commissioning groups. There are no plans to conduct a national review of hydrotherapy provision.

10 Dec 2015 | Written questions | Answered | House of Commons | 19246

Muscular Dystrophy: Hydrotherapy

Asked by: Gillan, Mrs Cheryl

To ask the Secretary of State for Health, if he will take steps to improve access for patients with muscular dystrophy to hydrotherapy services.

Answering member: George Freeman | Department of Health

NHS England is responsible for commissioning specialised neurological services, which includes services for patients with neuromuscular disorders. NHS England has published a service specification for neurological care, which sets out what providers must have in place to offer evidence-based, safe and effective services. The specification can be found at the following link:

www.england.nhs.uk/wp-content/uploads/2013/06/d04-neurosci-spec-neuro.pdf

Specialised neuromuscular care may include referral to local physiotherapy, hydrotherapy or rehabilitation if appropriate, however the commissioning of hydrotherapy services is a matter for local clinical commissioning groups. There are no plans to conduct a national review of hydrotherapy provision.

10 Dec 2015 | Written questions | Answered | House of Commons | 18884

Muscular Dystrophy: Drugs

Asked by: Lord Turnberg | **Party:** Labour Party

To ask Her Majesty's Government when they expect NICE to publish its opinion about the availability of Translarna for the treatment of patients with Duchene Muscular Dystrophy, following its approval by the European Medicines Agency in July 2014.

Answering member: Lord Prior of Brampton | **Party:** Conservative Party | **Department:** Department of Health

The National Institute for Health and Care Excellence (NICE) is the independent body that provides guidance on the clinical and cost effectiveness of drugs and treatments. NICE is currently evaluating Translarna (ataluren) for the treatment of Duchenne muscular dystrophy through its highly specialised technology programme. NICE currently expects to publish its final guidance in February 2016.

The NHS in England is legally required to fund drugs and treatments recommended in NICE highly specialised technology guidance within three months of its final guidance being issued. In the absence of guidance from NICE, it is for commissioners to make decisions on whether to fund medicines based on an assessment of the available evidence.

01 Dec 2015 | Written questions | Answered | House of Lords | HL3823

Muscular Dystrophy: Drugs

Asked by: Shannon, Jim

To ask the Secretary of State for Health, if he will make Translarna available on the NHS to treat Duchenne Muscular Dystrophy.

Answering member: George Freeman | Department of Health

The National Institute for Health and Care Excellence (NICE) is evaluating Translarna (ataluren) for the treatment of Duchenne muscular dystrophy through its highly specialised technology programme. NICE currently expects to publish its final guidance in February 2016.

The National Health Service in England is legally required to fund drugs and treatments recommended in NICE highly specialised technology guidance within three months of its final guidance being issued. In the absence of guidance from NICE, it is for commissioners to make decisions on whether to fund medicines based on an assessment of the available evidence.

23 Nov 2015 | Written questions | Answered | House of Commons | 16534

Muscular Dystrophy

Asked by: Campbell, Mr Gregory

To ask the Secretary of State for Health, what steps he is taking to promote research into muscular dystrophy conditions.

Answering member: George Freeman | Department of Health

The Department's National Institute for Health Research (NIHR) welcomes funding applications for research into any aspect of human health, including muscular dystrophies. These applications are subject to peer review and judged in open competition, with awards being made on the basis of the importance of the topic to patients and health and care services, value for money and scientific quality.

Research on novel approaches to treating neuromuscular disease including Duchenne muscular dystrophy is being carried out by the NIHR Biomedical Research Centre at Great Ormond Street Hospital for Children NHS Trust and the University College London Institute of Child Health.

The NIHR Health Technology Assessment programme is currently seeking to commission research on the effectiveness and cost-effectiveness of mechanical insufflation-exsufflation devices when compared to other methods of sputum clearance commonly used in children and young people with neuromuscular disease.

16 Sep 2015 | Written questions | Answered | House of Commons | 9997

Muscular Dystrophy: Drugs

Asked by: Cunningham, Mr Jim

To ask the Secretary of State for Health, what representations he has received from charities and patient groups on access to the drug Translarna for the treatment of Duchenne muscular dystrophy; and if he will make a statement.

Answering member: George Freeman | Department of Health

I met with the parents of children with Duchenne Muscular Dystrophy and Muscular Dystrophy UK, alongside the hon. Member for Winchester (Steve Brine) on 8 September 2015.

I also met with the Muscular Dystrophy Campaign (MDC) alongside the hon. Member for Leeds North West (Greg Mulholland) on 26 March 2015.

The former Minister of State for Care Services (Norman Lamb) met with the hon. Member for Blaydon (David Anderson) and a representative of the MDC group on 2 March 2015 where the issue of funding for Translarna was raised.

11 Sep 2015 | Written questions | Answered | House of Commons | 9650

Muscular Dystrophy: Drugs

Asked by: Lord Turnberg

To ask Her Majesty's Government whether they will make Translama available for the treatment of NHS patients with Duchenne muscular dystrophy as a matter of urgency.

Answering member: Lord Prior of Brampton | Department of Health

NHS England published its investment decisions for certain specialised services on 2 July 2015.

NHS England has agreed with the recommendations from NHS England's Clinical Priorities Advisory Group that a final funding decision on Ataluren (Translarna) for Duchenne muscular dystrophy should be made after the National Institute for Health and Care Excellence (NICE) has concluded its Highly Specialised Technology Appraisal process.

NICE is expected to produce draft guidance on Ataluren (Translarna) in October, with final guidance to follow shortly after. The European medicines regulator has also asked the manufacturer to undertake further studies of the treatment for completion in October.

There is also a process which allows for the consideration of critically clinically urgent cases falling outside of routine commissioned provision.

27 Jul 2015 | Written questions | Answered | House of Lords | HL1761

Engagements

Asked by: Caroline Nokes (Romsey and Southampton North) (Con)

Seven-year-old Jagger Curtis from Romsey suffers from Duchenne muscular dystrophy. Every day that he waits for first NHS England and now the National Institute for Health and Care Excellence to make a decision about Translarna is a day that threatens his mobility. Last week's decision to delay, potentially for up to five months, was a bitter blow. What action can my right hon. Friend take to make sure that NICE makes that decision with the utmost speed?

Answered by: The Prime Minister |

My hon. Friend rightly raises this issue, and I say to her that these are incredibly difficult decisions and we know how hard they are for patients and their families. I think it is right that it is expert clinicians at NHS England and not politicians who make these funding decisions, based on the available evidence. As she knows, NICE has not yet made a final decision on these drugs, so patients and their families, and other experts, can feed into its evidence-gathering and consultation process. She asks what we can do, and I think there are two things. First, when we have these drugs that cost over £400,000 per patient per year, it is right to ask some pretty challenging questions of the companies concerned and we should do so. Secondly, we must keep investing in our rare disease research and in genomics, and making sure that the NHS takes up these treatments rapidly. That is the sort of health service we want to build.

08 Jul 2015 | Oral questions - 1st Supplementary | Answered | House of Commons | House of Commons chamber | 598 c314

Muscle-wasting Conditions

Asked by: Harry Harpham (Sheffield, Brightside and Hillsborough) (Lab)

What steps he is taking to ensure that clinical commissioning groups routinely fund cough assist machines for people with muscle-wasting conditions when a clinical need has been identified.

Answering member: The Parliamentary Under-Secretary of State for Life Sciences (George Freeman) | Health

Muscle-wasting conditions associated with neurodegenerative disorders affect about 60,000 people in England at the moment. The Government are supporting research through the National Institute for Health Research, totalling £39 million. NHS England, CCGs and Muscular Dystrophy UK have come together and are jointly working on the "Bridging the Gap" report to improve neuromuscular disease, and the Department of Health is supporting this work with funding of £600,000. Decisions on the funding of cough-assist machines are rightly the responsibility of CCGs on a case-by-case basis.

Asked by: Harry Harpham

As revealed in Muscular Dystrophy UK's "Right to breathe" report published in February 2015, in some areas of the country patients have access to cough-assist machines which the local clinical commissioning group will not fund in other areas, despite a clinical need being clearly identified. These machines can help to prevent potentially fatal respiratory problems and to reduce costs and lengthy, unplanned hospital visits. A cough-assist machine costs £4,500, whereas a long stay in an intensive care unit can cost more than £13,000. [Interruption.] Will the Minister meet me and representatives of Muscular Dystrophy UK to discuss how better consistency in provision of vital respiratory equipment—

Answered by: George Freeman | Health

My answer of a few moments ago stands. Decisions on the commissioning of those machines are taken on a case-by-case basis locally. The National Institute for Health and Care Excellence has set out in guidance that cough-assist machines may be appropriate for some patients, but not in every area.

07 Jul 2015 | Oral answers to questions | House of Commons | House of Commons chamber | 598 c163

Engagements

Asked by: Mary Glindon (North Tyneside) (Lab)

Six young boys with the devastating disease of muscular dystrophy will be in Downing Street this afternoon, supported by Muscular Dystrophy

UK, to make a plea to the Prime Minister to help them access the Duchenne drug Translarna that they need now to stop them losing their mobility. Will the Prime Minister make time to see them and will he tell the House that these children can expect the positive answer they so desperately need now?

Answered by: The Prime Minister

I thank the hon. Lady for raising this issue. Muscular dystrophy is a terrible disease and I hugely admire the courage shown by the sufferers and their families. Unfortunately, I will not be able to hold that meeting this afternoon because I have to go from the statement after Prime Minister's questions straight to an EU summit in Brussels. I do remember meeting Archie Hill, who is one of the group, back in January. He is an amazing young boy—incredibly brave. The situation is that NHS England has now completed a consultation on how it prioritises investment in these specialised services, including drugs for rare conditions. It closed at the end of April and a decision can be expected in the near future. I recognise how vital it is to give those affected and their families a decision as soon as possible.

10 Jun 2015 | Oral questions - 1st Supplementary | Answered | House of Commons | House of Commons chamber | 596 c1184

Translarna (Duchenne Muscular Dystrophy)

Asked by: Caroline Nokes (Romsey and Southampton North) (Con)

When he expects NHS England to reach a decision on access to Translarna for the treatment of Duchenne muscular dystrophy; and if he will make a statement.

Answered by: The Parliamentary Under-Secretary of State for Health (Ben Gummer)

NHS England is considering the interim commissioning position for Translarna as part of its wider prioritisation process for funding in 2015-16 and expects to come to a decision by the end of this month. Translarna has also been referred for evaluation by the National Institute for Health and Care Excellence's highly specialised technologies programme. Draft NICE guidance will be available later this year, with final guidance expected in February 2016.

02 Jun 2015 | Oral questions - Lead | Answered | House of Commons | House of Commons chamber | 900001 | 596 cc437-9

Asked by: Caroline Nokes

I thank the Minister for that response and welcome him to his place. Yesterday my constituent Jules Geary came to see me regarding her son Jagger, who suffers from Duchenne muscular dystrophy. Jagger had been approved for Translarna treatment but then suddenly found that it had been withdrawn at the last moment. Like many other boys, he is now waiting, not knowing when a treatment that will prolong his mobility will be forthcoming. Will my hon. Friend meet me, Jules and

Muscular Dystrophy UK to discuss how this process can be streamlined so that other children do not have to wait this long?

Answered by: Ben Gummer | Health

Muscular dystrophy is a terrible, debilitating illness and my sympathies go out to Jagger and his family. My hon. Friend will be aware that families and their representatives will be going to Downing Street on 10 June to make their representations on this matter. The Minister for Life Sciences has introduced an accelerated access review precisely because of the concerns that my hon. Friend has raised, and I know that he will welcome representations once it has been completed.

Asked by: Mr Ronnie Campbell

Is the Minister aware of the case of my constituent, little George Pegg? At one time he could not walk, but this drug has made his life 100% better and he can now walk. Why are we dithering? This has been going on for at least a year, so why don't you get off that backside of yours and get it approved?

Answered by: Ben Gummer | Health

I thank the hon. Gentleman for his question. In relation to posteriors, it is good to see his in its rightful place. I have heard of his constituent's case, which is as distressing as that of Jagger and of all those suffering from Duchenne muscular dystrophy. It is a terrible disease that causes lasting pain to the sufferers and their families. That is precisely why we are pushing hard for a decision from NHS England by the end of this month—it could not have come as quick as he had hoped—and for interim NICE guidance by the end of this year. I am pushing officials to move as quickly as they can on this.

Asked by: Greg Mulholland (Leeds North West) (LD)

The reality is that NHS England has failed to respond to letters or to turn up for meetings, and it has behaved in an utterly unaccountable manner in regard not only to Translarna but to Vimizim, which is used to treat Morquio syndrome. We have still not had confirmation that an interim decision will be made on 25 June, but we are now being told that there will be a decision from NICE on 5 June. Will Ministers finally get a grip on this and give the families affected by these various conditions some sense of when they might get the treatment that could improve their quality of life?

Answered by: Ben Gummer | Health

I am sorry to hear that the hon. Gentleman has had that experience with NHS England. My hon. Friend the Minister for Life Sciences will want to speak to him about that; if it is the case, it is clearly unacceptable. As the hon. Gentleman will have heard from my previous answer, we are hoping to get quick decisions from NHS

England on the interim commissioning guidance this month, and I am pushing hard for a decision from NICE as soon as possible this year, so that we can get interim guidance from it.

Muscular Dystrophy

Asked by: Lord Taylor of Warwick

To ask Her Majesty's Government what steps they are taking to ensure that the National Health Service supports people with muscular dystrophy.

Answering member: Earl Howe | Department of Health

Through the Mandate we have asked NHS England to make measurable progress towards making our health service among the best in Europe at supporting people with long-term conditions such as muscular dystrophy, to live healthily and independently, with much better control over the care they receive.

NHS England commissions specialised neurological services at a national level, including for patients with muscular dystrophy. The neurosciences service specifications set out that patients with neuromuscular conditions, such as muscular dystrophy should have access to a multidisciplinary team (MDT) who will assess, diagnose and provide support. The MDT team will include neuromuscular consultants, neuromuscular physiotherapists, specialist nurses, occupational therapists, speech and language therapists and other care professionals. One of the key service outcomes of the specification is that all patients with long-term neurological conditions have an individualised care plan.

26 Mar 2015 | Written questions | Answered | House of Lords | HL5920

Topical Questions

Asked by: Greg Mulholland

I thank my right hon. Friend for that answer. I urge him to intervene in the campaign to get the drugs that are needed for those with Morquio syndrome, Duchenne muscular dystrophy and tuberous sclerosis. The Prime Minister said that there should be continuity of treatment, yet we have found out that that will not be delivered by the Department of Health. Katy Brown, the mother of my six-year-old constituent Sam Brown, has said that that is at best "misleading, at worst underhand". This situation is disgraceful. We need to fund those drugs now on an interim basis. Will my right hon. Friend speak to the Prime Minister and get it sorted this week?

Answered by: The Deputy Prime Minister

I pay tribute to my hon. Friend for the way he has sought to represent his constituent Sam Brown, and all the other children and their families who are—quite understandably—concerned about the continued provision of these drugs. As he heard from the Prime Minister when he raised the matter at Prime Minister's questions two weeks ago, the understanding is that NHS England is conducting a review that will conclude by the end of next month. In the meantime, drug companies will continue with the provision of these drugs until the end of May, so

that continuity is assured. Given my hon. Friend's concerns, I will undertake to look urgently at the matter again.

24 Mar 2015 | Oral questions - 1st Supplementary | Answered | House of Commons | House of Commons chamber | 594 c1277

Neuromuscular Disorders

Asked by: Anderson, Mr David

To ask the Secretary of State for Health, what plans he has to develop more regional managed clinical neuromuscular networks.

Answering member: Norman Lamb | Department of Health

Since 1 April 2013, NHS England has been responsible for commissioning specialised neurological services, which includes services for patients with neuromuscular disorders. NHS England has published a service specification for neurological care, which sets out what providers must have in place to offer evidence-based, safe and effective services. The specification can be found at the following link:

www.england.nhs.uk/wp-content/uploads/2013/06/d04-neurosci-spec-neuro.pdf

Specialised neuromuscular care may include referral to local or specialised physiotherapy, hydrotherapy or rehabilitation if appropriate. Provision of hydrotherapy services is a matter for the local National Health Service and may be accessed by patients with neuromuscular and other long-term debilitating conditions, subject to assessment and referral.

Neuromuscular patients may also be referred for enabling equipment such as wheelchairs adaptations and environmental controls in line with their clinical commissioning group or specialist rehabilitation referral criteria, subject to the complexity of need.

NHS England is currently undertaking a review of wheelchair services, led by Rosamond Roughton, National Director of Commissioning Development, which will consider provision across both specialised and non-specialised wheelchairs.

NHS England has set up strategic clinical networks (SCNs) for neurological conditions to provide clinical expertise and guidance. Alongside SCNs, Operational Delivery Networks (ODNs) are working with commissioners, providers and patients to ensure the delivery of safe and effective services across the patient pathway and help secure the best outcomes for all people with neurological conditions. Providers are at liberty to set up an ODN for neuromuscular services if they consider it would benefit service provision locally.

09 Jan 2015 | Written questions | Answered | House of Commons | 219448

3.2 Debates

Cystic Fibrosis

HC Deb | 603 cc2555-279WH

Drugs: Ultra-rare Diseases

HC Deb | 597 cc29-55WH

3.3 EDMs

MUSCULAR DYSTOPHY UK REPORT ON ACCESS TO HYDROTHERAPY

That this House welcomes the new report by Muscular Dystrophy UK on the urgent need to increase access to hydrotherapy across the UK; notes that many health professionals believe it improves or maintains mobility, strength and flexibility and is a good form of aerobic exercise for people with muscle-wasting conditions; expresses concern that individuals and families living with muscle-wasting conditions are being turned away from some hydrotherapy pools after being told they are only for use by people with broken bones; further notes that there are more than twice the number of hydrotherapy pools available for animals compared to those available for people; recognises the need for reasonable adjustments to be made to community swimming pools to ensure that they are accessible for people with muscle-wasting conditions; calls on the NHS to ensure that everyone with a muscle-wasting condition who requires hydrotherapy receives a referral; and further calls for a national review of hydrotherapy provision to be undertaken by the Department for Health.

03 Dec 2015 | Early day motions | Open | House of Commons | 817 (session 2015-16)

Primary sponsor: Glindon, Mary | **Party:** Labour Party

ACCESS TO DUCHENNE MUSCULAR DYSTROPHY TREATMENT, TRANSLARNA

That this House notes the draft guidance published by NICE to not approve the Duchenne muscular dystrophy treatment called Translarna at this stage; acknowledges that NICE has requested further data on cost and information from the latest phase of the clinical trial from PTC Therapeutics, the pharmaceutical company which manufactures the drug; recognises that a boy in Scotland affected by Duchenne muscular dystrophy will shortly receive Translarna after an individual funding request to the local NHS Board; expresses concern from Muscular Dystrophy UK and the Duchenne community that families whose children could benefit from the treatment have waited over a year since European Commission approval for Translarna; further recognises that

boys can only take the drug if they are aged five and over and still walking; further notes that Translarna is available to patients in several European countries; and calls on NICE to urgently reach a positive decision at the next stage of the process.

19 Oct 2015 | Early day motions | Open | House of Commons | 530 (session 2015-16)

Primary sponsor: Glindon, Mary | **Party:** Labour Party

WORLD DUCHENNE AWARENESS DAY

That this House recognises that World Duchenne Awareness Day on 7 September 2015 offers the opportunity to create greater knowledge of that condition; offers congratulations to Laura Smith, a mother from Limavady, who was declared the Muscular Dystrophy UK champion for her outstanding work in highlighting the incurable form of muscular dystrophy known as Duchenne which her six-year-old son Callum suffers from; and commends her and all those who campaign for more research into the condition.

08 Sep 2015 | Early day motions | Open | House of Commons | 409 (session 2015-16)

Primary sponsor: Campbell, Gregory | **Party:** Democratic Unionist Party

NHS ENGLAND BUREAUCRACY

That this House expresses extremely strong concern that despite 14 months having passed since the treatment Vimizim was licensed by the European Medicines Agency in April 2014, it remains unavailable for use through NHS England; notes a series of broken commitments on decision dates, moved from 25 June 2015 to 30 June 2015 to 1 July 2015 to eventually 2 July 2015; further notes NHS England's eventual decision was to delay making a decision until October 2015; believes NHS England ultimately failed to make a decision on access to the treatments Vimizim and Translarna, while those who need Everolimus treatment remain in the dark about the possibility of a prescribing policy; strongly believes that it is unacceptable that 218 children and young adults with Morquio syndrome, Duchenne muscular dystrophy and tuberous sclerosis complex are being repeatedly let down and suffering the consequences of bureaucracy; and believes that those with ultra-rare progressive conditions do not have the luxury of time and need immediate access to these licensed life-changing treatments.

07 Jul 2015 | Early day motions | Open | House of Commons | 264 (session 2015-16)

Primary sponsor: Mulholland, Greg | **Party:** Liberal Democrats

4. Further reading

Further information about muscular dystrophy and its treatment can be found on the following pages.

Muscular dystrophy - Treatment , NHS Choices website

The Muscular Dystrophy Campaign

Action Duchenne

DEBATE PACK
Number CDP-0130
14 December 2015

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